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DECREASING GENE EXPRESSION IN A MAMMALIAN SUBJECT IN VIVO VIA AAV-MEDIATED RNAi EXPRESSION CASSETTE TRANSFER

Abstract

Decreasing the expression of genes in a mammalian subject has multiple applications ranging from cancer therapy to anti-infective therapy or treatment of autosomal dominant genetic disorders. Yet, there is still a lack of efficient technologies to achieve that goal in mammalian subjects in vivo. The present invention relates to methods for decreasing gene expression by administering to a mammalian subject a recombinant adeno-associated viral vector in vivo with said vector comprising an RNA interference (RNAi) expression cassette whose RNA expression products directly or indirectly lead to a decrease in expression of the corresponding RNAi target gene. Upon successful transduction with the recombinant adeno-associated viral vector, the RNA expression products of the RNAi expression cassette will decrease the cellular concentration of the mRNA transcripts of the RNAi target gene,

thus resulting in decreased concentration of the protein encoded by the RNAi target gene.